

### September 2015 Newsletter

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# CIRM-Funded Clinical Trial for Spinal Cord Injury Reports Promising Results

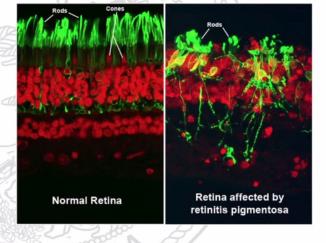


On August 31st, Asterias Biotherapeutics reported preliminary positive results from its CIRM-funded clinical study using a human embryonic stem cell-based therapy to treat patients with spinal cord injury. In the first part of the Phase 1/2a clinical trial, three patients with severe spinal cord injuries at the neck were given a "low dose" injection of two million so-called AST-OPC1 cells to test the safety and feasibility of the stem cell treatment. The AST-OPC1 cells give rise to cells responsible for forming a conductive sheet around nerve cells that allows nerves to send electrical signals from one nerve to another. At the two month post-injection assessment, the first patient experienced no side effects and had an improved ASIA score, an impairment injury assessment scale. The other two patients showed no side effects. Asterias plans to treat a second group of patients with higher doses of AST-OPC1 cells.

- Read our blog about the Asterias report
- Learn more about CIRM-funded spinal cord injury research

#### **CIRM-Funded Clinical Trial for Blinding Disease Treats First Patients**

# CIRM Spotlight on Retinitis Pigmentosa and Stem Cell Research





presented by **Henry Klassen University of California, Irvine** 



On August 12th, a CIRM-funded, UC Irvine-led clinical trial team announced they had treated their first four patients with a cell-based therapy for retinitis pigmentosa, an incurable disease that leads to blindness. The patients were each given a single injection of retinal progenitor cells in the most affected eye. It's hoped these cells will help protect the photoreceptors in the retina that have not yet been damaged by RP, and even revive those that have been damaged but not yet destroyed by the disease. Sixteen patients will be enrolled in this first phase and will be followed for 12 months to determine if the therapy is safe and if it has any beneficial effects on vision compared to the untreated eye. To learn more about the study or to enroll contact the UCI Alpha Stem Cell Clinic at 949-824-3990 or by email at stemcell@uci.edu.

- · Read our press release and blog about this milestone
- Watch a presentation from team leader, Henry Klassen, about the trial

#### California's Stem Cell Bank is Open for Business

As part of the iPSC Initiative, CIRM is asking for people like you to contribute blood or skin samples that can be used to make induced pluripotent stem cells. These cells would then be made available to researchers throughout California and the rest of the world.

# How does the donation process work?

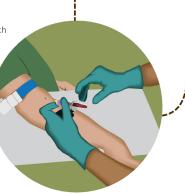
1. Researchers will ask

their patients for permission to use their cells and health information for the stem cell bank. This process of getting permission is called *informed consent*, during which patients and/or their guardians will receive detailed information about the research.



2. A clinic will collect

a blood (or skin) sample along with information about your health. All information identifying the sample as yours will be removed and replaced by a code to protect your privacy. Information about the sample (such as whether it comes from a healthy individual, or from someone with a condition such as autism or Alzheimer's disease) will be used to describe the eventual iPS cells. This information allows researchers using these iPS cells to design effective experiments.



# What happens to my donated sample?



3. The blood or skin sample will be transferred to a laboratory in Marin County, California, where it will be transformed into stem cells. The resulting stem cells will be placed in the stem cell bank along with their descriptive health information, and made available to scientists.

# How will the stem cell lines be used?

4. Qualified researchers in California

and the rest of the world will be able to request stem cells from the bank.
CIRM is creating the bank so scientists can use the cells, either in a dish or transplanted into animals, to:

- \* Study how disease develops and progresses, and
- \* Test new drugs or other treatments.

The large size of the collection is designed to help scientists understand how disease and treatment may vary in a diverse population like California's.



On September 1st, Coriell Institute for Medical Research opened the world's largest publicity available stem cell bank. The CIRM-funded Bank is offering the first 300 different induced pluripotent stem cell (iPSC) lines for researchers interested in gaining a deeper understanding of, and developing treatments for, 11 common diseases and disorders such as autism, heart, liver, lung and eye disease. Tissue samples are being collected by researchers at UC San Francisco, UC San Diego, UC Los Angeles and Stanford University. All donors undergo a rigorous consent and approval process before any tissue is collected. Once collected, those samples are then turned into different cell lines by Cellular Dynamics International (CDI) and then stored and distributed by Coriell Institute from a facility at the Buck Institute in Novato, California. Ultimately, tissue samples from up to 3000 volunteer donors will be collected.

- Read our press release about the Bank
- Learn more about how to buy a cell line
- Read a brochure about the Bank

High Schoolers Celebrate a Stem Cell Summer of Taylor Swift and Jay-Z



Through the support of CIRM's Creativity Awards grant, about 70 high school students across California spent their summer getting hands-on experience doing stem cell research at nine universities in the state. On August 7th, CIRM hosted Creativity Day in San Mateo which showcased the accomplishments of the students. The remarkable, young students gave graduate-level research presentations and showed off posters of their scientific findings to their lab mentors, the CIRM team, and proud family members. As part of their internship, students also submitted blogs and instagram photos about their time in the lab. And a few groups produced fabulous, inventive videos that parodied popular songs by the likes of Taylor Swift, Jay-Z and Carly Rae Jepsen. Over the course of the three-year CIRM Creativity Awards program, our agency has supported 220 high school students in stem cell research internships.

- Read our blog about Creativity Day 2015
- Watch all the Creativity Day video submissions
- Read the winning Creativity Day blog submission

#### The Latest Publications by CIRM-Funded Researchers

CIRM grantees are making steady progress on understanding the fundamentals of stem cell biology and moving stem cell-based therapies toward clinical trials. Here's an example of CIRM-funded research that was published in August along with a press release from the grantees' home institution and an entry from *The Stem Cellar*, CIRM's official blog:

# August 24th - Cell Stem Cell

- UCSD Release: Study Provides Hope for Some Human Stem Cell Therapies
- CIRM Blog: Study Identifies Safer Stem Cell Therapies

# **Upcoming CIRM Events**

#### September 24th: CIRM Board Meeting

The CIRM governing Board will hold a telephonic meeting on September 24th. Agendas and details will be posted to the **meetings page** 10 days before each event.

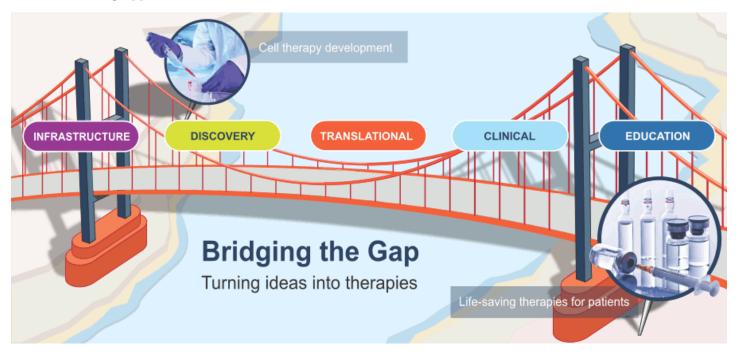
#### October 7th-9th: Stem Cell Meeting on the Mesa

The Stem Cell Meeting on the Mesa is a three-day conference bringing together senior executives and top decision-makers in the regenerative medicine and advanced therapies industry with the scientific community to advance cutting-edge research into cures. CIRM President and CEO, C. Randal Mills, and several CIRM-grantees are scheduled to speak at the event.

#### October 14th: Stem Cell Awareness Day

October 14th marks Stem Cell Awareness Day 2015, which brings together organizations and individuals around the world working to ensure that we realize the benefits of one of the most promising fields of science in our time. The day is a unique global opportunity to foster greater understanding about stem cell research and the range of potential applications for disease and injury. Visit our stem cell awareness day page frequently for updates on the day's events.

# **CIRM 2.0 Funding Opportunities**



## Clinical:

- PA 15-01: Partnering opportunity for late stage preclinical projects
- PA 15-02: Partnering opportunity for clinical trial stage projects
- PA 15-03: Partnering opportunity for supplemental accelerating activities
- Visit our Clinical Trial Stage Programs Page for more information

#### **Education:**

- RFA 15-05: Bridges to Stem Cell Research and Therapy Awards program
- RFA 15-06: Summer Program to Accelerate Regenerative Medicine Knowledge (SPARK)

Our Discovery and Translational Stage Programs will be announced later this year.

Sign up to receive CIRM funding announcements

#### **CIRM Job Openings**

The following positions are now open. Follow the links below to apply or go to our jobs page:

- · Systems Engineer
- Science Officer/Senior Science Officer, Therapeutics
- · Associate Director/Director of Therapeutics
- · Grants Management Specialist